

Stories of Hope: The CIRM Stem Cell Four

At our 2016 CIRM December Board meeting, we invited four people to speak about their experience participating in CIRM-funded stem cell clinical trials. We're calling them the CIRM Stem Cell Four, and their inspirational stories remind us that the ultimate goal of funding stem cell research is to find treatments that can cure patients with unmet medical needs. We hope that the voices of these individuals will motivate others who are suffering from diseases that have no cures.

Brenden Whittaker



At the age of one, Brenden was diagnosed with X-linked chronic granulomatous disease (X-CGD), a rare, genetic disease that causes the immune system to malfunction leaving it unable to fight off certain bacterial and fungal infections which, over time, can be life-threatening. Hospitalized hundreds of times over the years Brenden almost died on a couple of occasions and had part of his lung and liver removed due to repeated infection.

On December 13, 2015, Brenden was cured thanks to an experimental procedure, developed by Dr. Don Kohn, that removed some of his own blood stem cells; they were then genetically modified to remove the gene that causes the problem, and then re-infused to him. The modified blood stem cells created a new blood system and a healthy immune system capable of fighting off infections.

Now that Brenden is cured of his disease, he is getting ready to go back to college full-time. "I want to get into pre-med, go to med school and become a doctor. All the experience I've had has just made me more interested in being a doctor, I just want to be in a position where I can help people going through similar things to what I experienced."

Evangelina Padilla-Vaccaro



Evangelina was diagnosed shortly after birth with severe combined immunodeficiency (SCID); it's also known as "bubble baby" disease because in the past, children were kept in a sterile plastic bubble to protect them from getting sick. It's a rare – and often deadly -- genetic disorder that meant she had no functioning immune system, leaving her vulnerable to infections. Many children with this condition die within the first year of life.

Evangelina was cured as part of a clinical trial at UCLA run by Dr. Don Kohn. She underwent a stem cell transplant that took her own blood stem cells, genetically re-engineered them, and returned them to her body. These re-engineered stem cells created a new blood and immune system.

Angelina can now play outside and attend parties with her twin sister Annabella. Her parents Alysia and Christian are overjoyed that both their children are healthy. "I get to look at my kids the way other parents should be looking at their children all the time, but until you are put in that position you never appreciate it, says Alysia. "We take things for granted. We all do. I know I do, particularly when the twins drive me crazy, but I also know how very precious this is."

Alysia could not hold back her emotions at the December Board meeting and said through tears, "Thank you for keeping my family complete."

Read more about Evangelina's story on the Stem Cellar blog.

Jake Javier



An accident at a friend's pool party on the eve of Jake's high school graduation left him paralyzed from the chest down with no ability to use either his arms or hands.

On Thursday, July 7th 2016, a few weeks later, Jake Javier became a member of a very select group. Jake underwent a stem cell transplant being tested in a CIRM-funded clinical trial sponsored by Asterias Biotherapeutics. He had 10 million stem cells transplanted into his neck at Santa Clara Valley Medical Center in San Jose. Since then Jake has regained use of his arms and is working hard to hopefully regain movement in other parts of his body.

"I've tried hand-cycling, using my hands and arms to power the bike rather than my feet. I liked that and might be interested in getting one of those bikes, could be useful for getting around. And I've been doing things I've never done before, like scuba diving. I got help putting on the gear and then went to the bottom of a 12 foot deep swimming pool. It was really cool. I liked that a lot."

Even as he works hard on regaining the use of his arms and hands, he has an eye on the future: "Next fall I intend going back to school, to Cal Poly. I'm signed up for mechanical engineering. In the meantime I'm going to try and enroll in some online classes to help get back into the swing of things."

You can read more about Jake's story on our Stem Cellar Blog [here](#) and [here](#).

Karl Trede



In 2006, Karl was diagnosed with throat cancer. He had the tumor and his vocal cords removed and now relies on a voice box to speak. Several years later, doctors found the cancer had come back and this time had spread to his lungs. This is how Karl became patient #1 in a CIRM-funded clinical trial at Stanford.

"One day he (the doctor) said we have a new trial we're going to start would you be interested. I said "sure". I don't believe I knew at the time that I was going to be the first one but I thought I'd give it a whirl. Fortunately, I lasted 13 months, 72 treatments with absolutely no side effects."

The trial used a monoclonal antibody to target a specific protein, called CD47, that sits on the surface of cancer cells. This protein tricks the immune system into not attacking the cancer, earning CD47 the nickname from the Stanford researchers as the "don't eat me" protein. The monoclonal antibody disables CD47, leaving the cancer vulnerable to being attacked by the patient's own immune system. The therapy has stopped Karl's cancer from growing, which has allowed him to spend more time with his wife, Vita, and family.

Karl told us that it was an easy choice for him to be the first patient in this trial. He said, "There's no effective treatment for this cancer. It's not likely, but it's possible this could be the one. If nothing else, and if it doesn't do anything for me, hopefully it does something so they learn for others."

People like Karl, Jake, Evangelina and Brenden and their families are the reason why stem cell research moves forward. Their brave efforts to advance stem cell treatments through clinical trials will help our agency attain our mission and hopefully one day, fund a treatment that develops into a cure.

**Special thanks to David Jensen for coining the term "California Stem Cell Four" and writing about these patients on his blog California Stem Cell Report.*

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